(19) World Intellectual Property Organization

International Bureau



(43) International Publication Date 19 May 2005 (19.05.2005)

PCT

(10) International Publication Number WO 2005/045035 A3

- (51) International Patent Classification⁷: C12N 15/11, C12P 19/34, C07H 21/02, 21/04, A61P 31/00, A61K 31/713
- (21) International Application Number:

PCT/US2004/026930

- (22) International Filing Date: 20 August 2004 (20.08.2004)
- (25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

| 10/693,059 | 23 October 2003 (23.10.2003) | US |
|----------------|-------------------------------|----|
| 10/720,448 | 24 November 2003 (24.11.2003) | US |
| 10/727,780 | 3 December 2003 (03.12.2003) | US |
| 10/757,803 | 14 January 2004 (14.01.2004) | US |
| 60/543,480 | 10 February 2004 (10.02.2004) | US |
| 10/780,447 | 13 February 2004 (13.02.2004) | US |
| 10/826,966 | 16 April 2004 (16.04.2004) | US |
| PCT/US04/13456 | 30 April 2004 (30.04.2004) | US |
| PCT/US04/16390 | 24 May 2004 (24.05.2004) | US |
| | | |

- (71) Applicant (for all designated States except US): SIRNA THERAPEUTICS, INC. [US/US]; 2950 Wildemess Place, Boulder, CO 80301 (US).
- (72) Inventors; and
- (75) Inventors/Applicants (for US only): MCSWIGGEN, James [US/US]; 4866 Franklin Drive, Boulder, CO 80301 (US). CHOWRIRA, Bharat, M. [US/US]; 576 Manorwood Lane, Louisville, CO 80027 (US). HAEBERLI, Peter [US/US]; 705 7th Street, Boulder, CO 80513 (US).

- (74) Agent: TERPSTRA, Anita, J.; McDonnell Boehnen Hulbert & Berghoff LLP, 300 South Wacker Drive, Suite 3200, Chicago, IL 60606 (US).
- (81) Designated States (unless otherwise indicated, for every kind of national protection available): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NA, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.
- (84) Designated States (unless otherwise indicated, for every kind of regional protection available): ARIPO (BW, GH, GM, KE, LS, MW, MZ, NA, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PL, PT, RO, SE, SI, SK, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

Published:

- with international search report
- before the expiration of the time limit for amending the claims and to be republished in the event of receipt of amendments
- (88) Date of publication of the international search report: 8 December 2005

For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.

(54) Title: RNA INTERFERENCE MEDIATED INHIBITION OF NOGO AND NOGO RECEPTOR GENE EXPRESSION USING SHORT INTERFERING NUCLEIC ACID (siNA)

(57) Abstract: This invention relates to compounds, compositions, and methods useful for modulating NOGO and/or NOGO receptor gene expression using short interfering nucleic acid (siNA) molecules. This invention also relates to compounds, compositions, and methods useful for modulating the expression and activity of other genes involved in pathways of NOGO and/or NOGO receptor gene expression and/or activity by RNA interference (RNAi) using small nucleic acid molecules. In particular, the instant invention features small nucleic acid molecules, such as short interfering nucleic acid (siNA), short interfering RNA (siRNA), double-stranded RNA (dsRNA), micro-RNA (miRNA), and short hairpin RNA (shRNA) molecules and methods used to modulate the expression of NOGO and/or NOGO receptor genes, such as NOGO-A, NOGO-B, NOGO-C, NOGO-66 receptor, NI-35, NI-220, NI-250, myelin-associated glycoprotein, tenascin-R, and NG-2.

